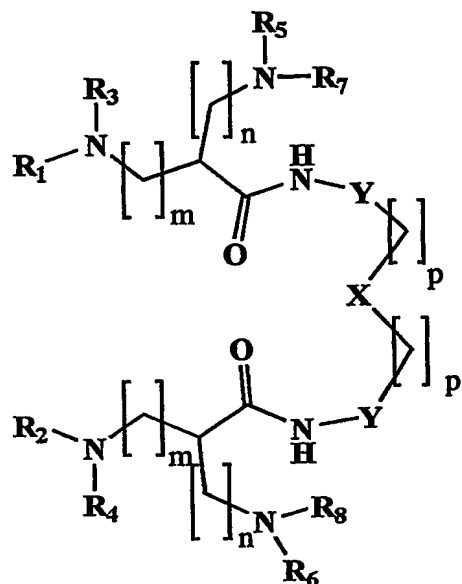


## Claims

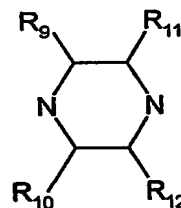
1. A diaminoacid-polyamine:peptide based gemini compound having a diaminoacid-polyamine or a diaminoacid-aminoacid-polyamine backbone and
- 5 conforming to the general structure of formula (I):



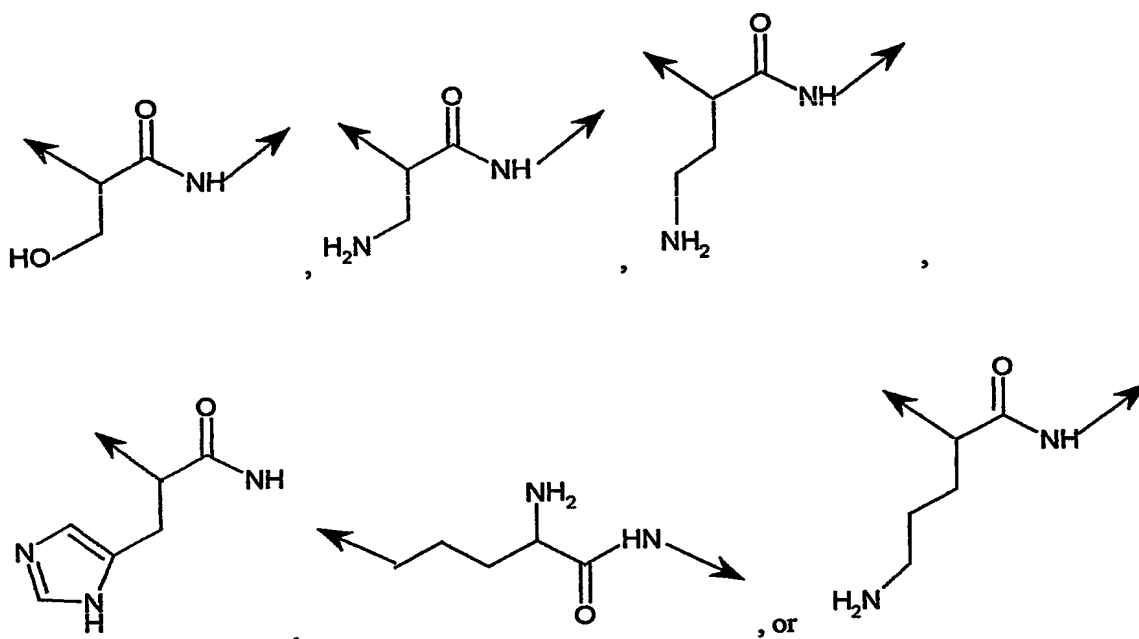
(I)

where:

- 10  $m = 0$  to  $6$ ;  
 $n = 0$  to  $7$ ;  
 $p = 0$  to  $6$ ; and where



- 15  $X =$  a bond,  $\text{CH}_2$ ,  $(\text{CH}_2)_2$ ,  $\text{NH}(\text{CH}_2)_q\text{NH}$  where  $q = 2$  to  $6$ , or where  $R_9$  to  $R_{12}$ , which can be the same or different, are selected from H, O or  $\text{C}_r\text{H}_{2r+1}$ , where  $r = 0$  to  $6$  with the proviso that when  $R_9$  and  $R_{12}$  are O, or when  $R_9$  and  $R_{11}$  are O, then  $R_{10}$  and  $R_{11}$  or  $R_{10}$  and  $R_{12}$ , respectively, are H; and where
- 20  $Y =$  a bond,  $\text{CH}_2$ .



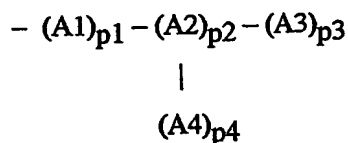
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and where  $R_3$ ,  $R_4$ ,  $R_5$ ,  $R_6$ ,  $R_7$  and  $R_8$  are hydrogen and  $R_1$  and  $R_2$  are saturated or unsaturated hydrocarboxyl groups having up to 24 carbon atoms and linked to the diaminoacid-polyamine backbone by an amide bond; or

10

where  $R_3$ ,  $R_4$ ,  $R_5$  and  $R_6$  are hydrogen,  $R_1$  and  $R_2$  are saturated or unsaturated hydrocarboxyl groups having up to 24 carbon atoms and linked to the diaminoacid-polyamine backbone by an amide bond, and where  $R_7$  and  $R_8$ , which may be the same or different, are peptide groups formed from one or more amino acids linked together by amide (CONH) bonds and further linked to the diaminoacid-polyamine backbone by amide bonds, in a linear or branched manner, having the general formula (II):

15



(II)

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where the values for  $p1$  and  $p2$ , which may be the same or different, are from 0 to 5, preferably 1;

and the values for  $p3$  and  $p4$ , which may be the same or different, are from 0 to 5, preferably 0;

$A1$ ,  $A3$  and  $A4$ , which may be the same or different, is an amino acid selected from serine, lysine, ornithine, threonine, histidine, cysteine, arginine and tyrosine; and

$A2$  is an amino acid selected from lysine, ornithine and histidine;

or

a pharmaceutically acceptable salt thereof..

2. A compound according to claim 1 that is symmetrical, that is  $R_1$  and  $R_2$  are the same as each other,  
 5  $R_3$  and  $R_4$  are the same as each other,  $R_5$  and  $R_6$  are the same as each other,  $R_7$  and  $R_8$  are the same as each other.

3. A compound according to claims 1 or 2 wherein A1 is lysine, serine or threonine, and A3 and A4 are lysine, ornithine, histidine or arginine.

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4. A compound according to any of claims 1 to 3 wherein the hydrocarboxyl group is selected from:

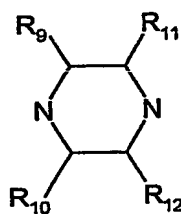
$-\text{C}(\text{O})(\text{CH}_2)_{10}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_{12}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_{14}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_{16}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_{18}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_{20}\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_5\text{CH}_3$  natural mixture  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_7\text{CH}_3$  natural mixture  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_5\text{CH}_3$  Cis  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_7\text{CH}_3$  Cis  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_5\text{CH}_3$  Trans  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CH}(\text{CH}_2)_7\text{CH}_3$  Trans  
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CH}=\text{CHCH}_2\text{CH}=\text{CH}(\text{CH}_2)_4\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_7(\text{CH}=\text{CHCH}_2)_3\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_3\text{CH}=\text{CH}(\text{CH}_2\text{CH}=\text{CH})_3(\text{CH}_2)_4\text{CH}_3$   
 $-\text{C}(\text{O})(\text{CH}_2)_7\text{CHCH}(\text{CH}_2)_7\text{CH}_3$   
 $-\text{C}(\text{O})\text{CHCHOH}(\text{CH}_2)_2\text{CH}_3$  or  
 $-\text{C}(\text{O})(\text{CH}_2)_{22}\text{CH}_3$ .

15

5. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is  $(\text{CH}_2)$  or  $(\text{CH}_2)_2$ , Y is a bond and p is 0 to 4.

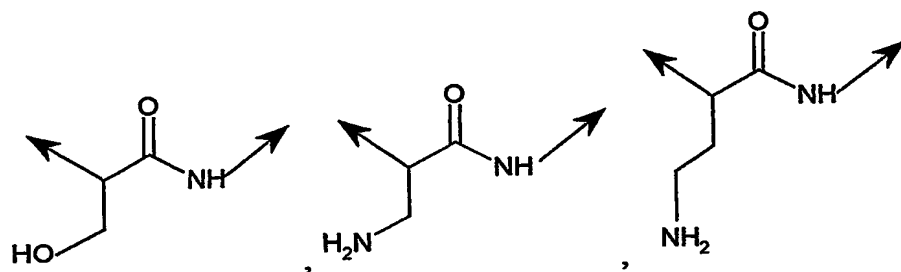
6. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is  
 20  $\text{NH}(\text{CH}_2)_q\text{NH}$ , where q is 2 to 5, Y is a bond and p is 2 to 5.

7. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is

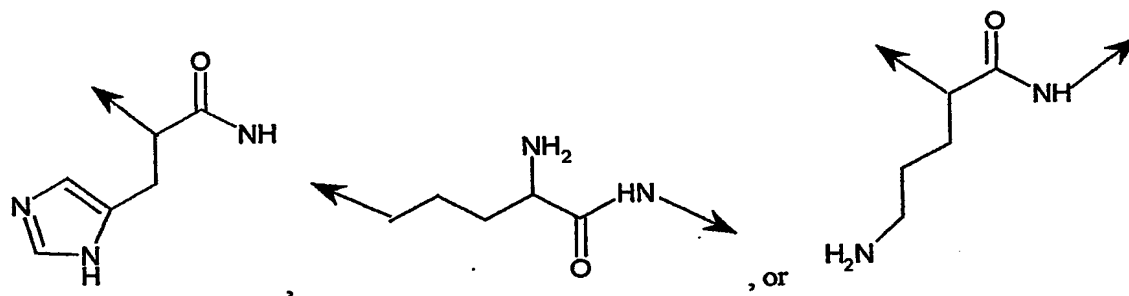


, where  $R_9$ ,  $R_{10}$ ,  $R_{11}$  and  $R_{12}$  are all H, Y is a bond and p is 2 to 5.

- 5 8. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is  $(CH_2)$  or  $(CH_2)_2$ , p is 0 to 4 and Y is

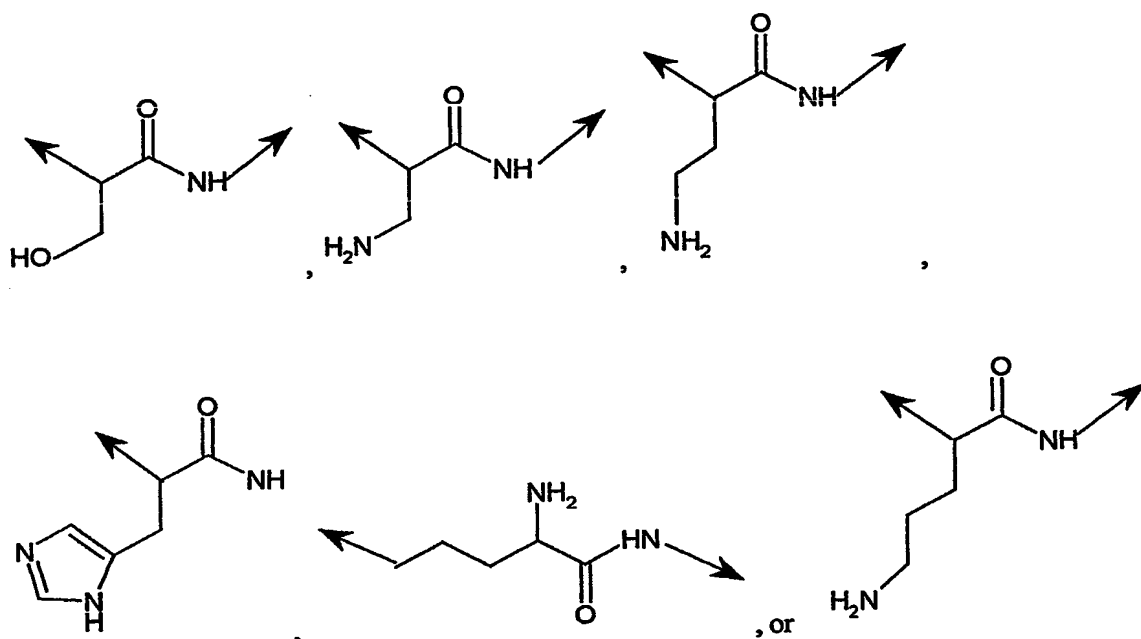


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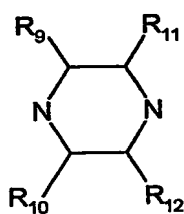
9. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is  $NH(CH_2)_qNH$ , where q is 2 to 5, p is 2 to 5 and Y is

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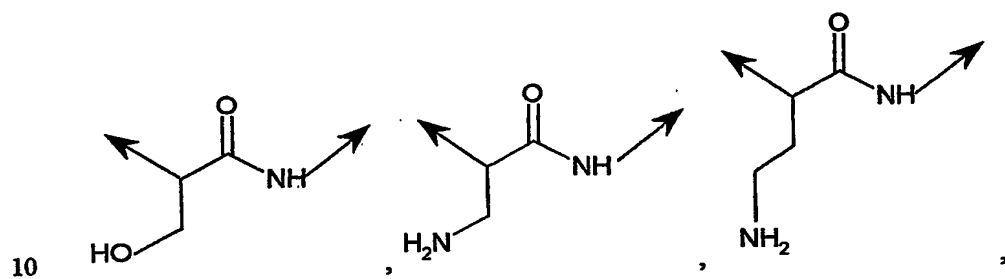


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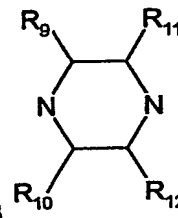
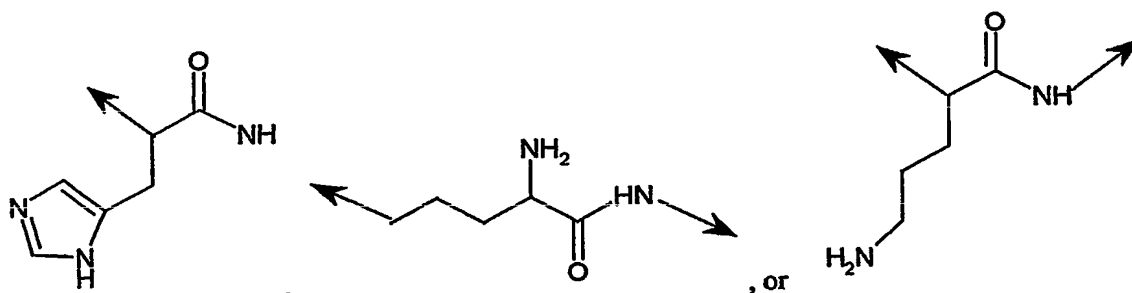
10. A compound according to any one of claims 1 to 4 where m is 0, n is 2 to 4, X is



, where  $R_9$ ,  $R_{10}$ ,  $R_{11}$  and  $R_{12}$  are all H, p is 2 to 5 and Y is



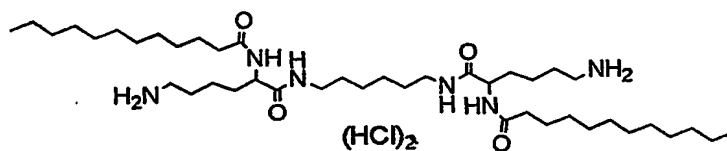
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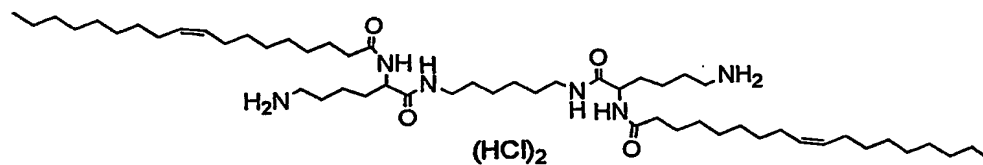
11. A compound according to any one of claims 1 to 4 where X is 1 to 6 and n is 1 to 7.

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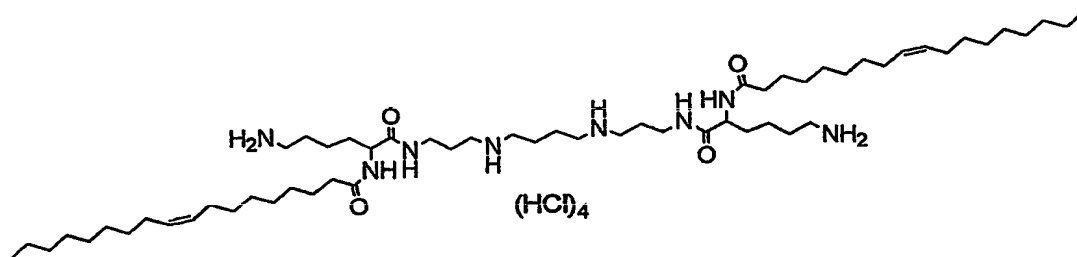
12. The compound GSN 11 of formula:



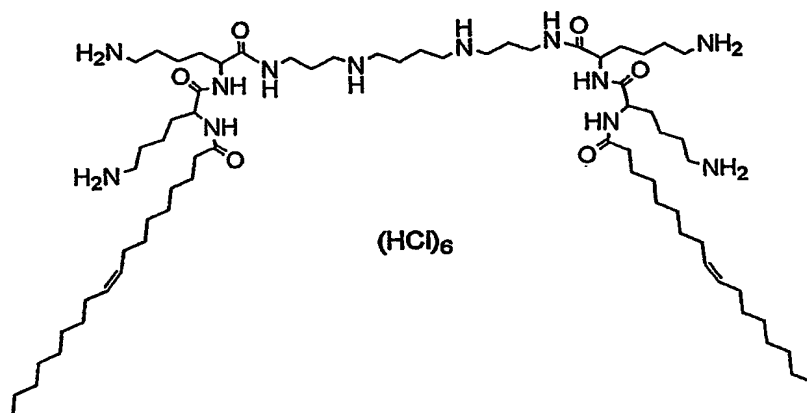
- 10 13. The compound GSN 14 of formula:



14. The compound GSC 102 of formula:

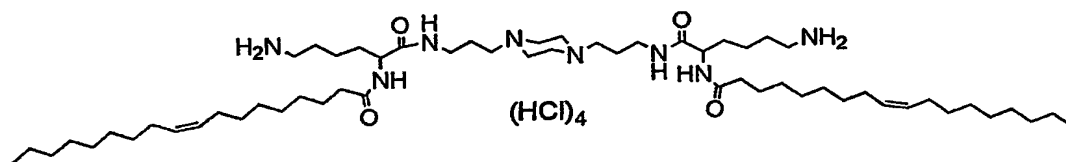


15. The compound GSC 157 of formula:



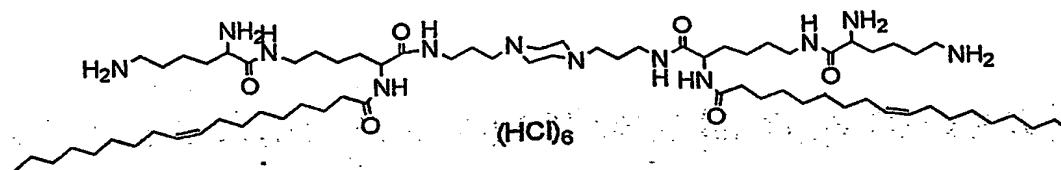
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16. The compound GSC170 of formula:

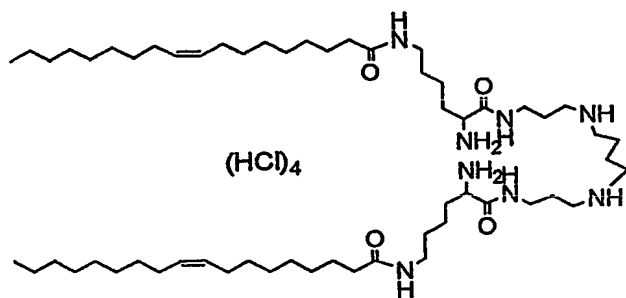


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17. The compound GSC 184 of formula:



- 15 18. The compound GSC101 of formula:



19. The use of a di-amino acid-polyamine-peptide-based gemini compound as defined in any one of claims 1 to 18 in enabling transfection of DNA or RNA or analogues thereof into a eukaryotic or prokaryotic cell *in vivo* or *in vitro*.
20. The use of a di-amino acid-polyamine-peptide-based gemini compound according to claim 19 wherein the compound is used in combination with one or more supplements selected from the group consisting of:
- (i) a neutral carrier; or
  - (ii) a complexing reagent.
21. The use according to claim 20 wherein the neutral carrier is dioleoyl phosphatidylethanolamine (DOPE).
22. The use according to claim 20 wherein the complexing reagent is PLUS reagent.
23. The use according to claim 20 wherein the complexing reagent is a peptide comprising mainly basic amino acids.
24. The use according to claim 23 wherein the peptide consists of basic amino acids.
25. The use according to claim 23 or 24 wherein the basic amino acids are selected from lysine and arginine.
26. The use according to claim 23 wherein the peptide is polylysine or polyornithine.
27. A method of transfecting polynucleotides into cells *in vivo* for gene therapy, which method comprises administering di-amino acid-polyamine-peptide-based gemini compounds of any one of claims 1 to 18



together with, or separately from, the gene therapy vector.

28. The use of a diaminoacid-polyamine-based gemini compound of any one of claims 1 to 18 to facilitate the transfer of a polynucleotide or an anti-infective compounds into prokaryotic or eukaryotic organism for use in anti-infective therapy.
29. The use of a diaminoacid-polyamine-based gemini compound of any one of claims 1 to 18 to facilitate the adhesion of cells in culture to each other or to a solid or semi-solid surface.
30. A process for preparing diaminoacid-polyamine-based gemini compounds of claim 1 which process comprises the coupling of a succinimide ester of a diaminoacid linked to its  $\alpha$  or terminal amino group to an hydrocarboxyl chain to a polyamine linker using potassium carbonate as a base in a mixture of tetrahydrofuran and water as solvents.